BELLUS Health Announces Positive Topline Results from its Phase 2b SOOTHE Trial of BLU-5937 for the Treatment of Refractory Chronic Cough

December 13, 2021

Primary efficacy endpoint statistically significant with 34% placebo-adjusted reduction in 24-hour cough frequency observed at 50 mg and 200 mg BID doses ($p \leq 0.005$)

BLU-5937 was well-tolerated with a low rate of taste-related adverse events (≤6.5%); treatment emergent adverse event profile comparable to placebo

Company also provides an update on its P2X3 pipeline and BLU-5937 Phase 2a trial for the treatment of chronic pruritus

Company to host conference call and webcast at 8:00 a.m. ET

LAVAL, Quebec--(BUSINESS WIRE)--Dec. 13, 2021-- BELLUS Health Inc. (Nasdaq:BLU; TSX:BLU) ("BELLUS Health" or the "Company"), a clinical-stage biopharmaceutical company developing novel therapeutics for the treatment of refractory chronic cough ("RCC") and other hypersensitization-related disorders, today announced that the 50 mg and 200 mg BID doses of BLU-5937 in its Phase 2b SOOTHE trial for the treatment of RCC achieved statistical significance on the primary endpoint with 34% placebo-adjusted reduction in 24-hour cough frequency observed ($p \leq 0.005$) at day 28. BLU-5937 was generally well-tolerated, with low rates of taste-related adverse events reported (≤6.5%) at all doses.

Mr. Roberto Bellini, Chief Executive Officer of BELLUS Health commented, "We are extremely pleased with the compelling topline results from the Phase 2b SOOTHE trial which highlight BLU-5937's best-in-class potential for the treatment of refractory chronic cough. RCC is a prevalent and growing condition that significantly impacts the quality of life of an estimated 9 million patients in the United States and 9 million patients in Europe."

Summary of Topline Results: Phase 2b SOOTHE Trial in Refractory Chronic Cough

Efficacy Results:

The SOOTHE trial, which enrolled 249 participants with a baseline awake cough frequency of ≥ 25 per hour, demonstrated a clinically meaningful and statistically significant placebo-adjusted reduction in 24-hour cough frequency of 34% at the 50 mg and 200 mg BID dose levels of BLU-5937 ($p \leq 0.005$) at day 28. The 12.5 mg BID dose demonstrated a statistical trend with 21% reduction in placebo-adjusted 24-hour cough frequency ($p=0.098$) with a dose response observed between the 12.5 mg and 50 mg BID doses.

SOOTHE Primary Efficacy Endpoint

Dose	Placebo-adjusted change in 24-hour cough frequency at day 28	p-value
12.5 mg BID	-21.1%	p=0.098
50 mg BID	-34.4%	p=0.003
200 mg BID	-34.2%	p=0.005

Safety and Tolerability Results:

BLU-5937's safety and tolerability data were consistent with previous trials, including the Phase 2a RELIEF trial. BLU-5937 was well-tolerated with low impact on taste perception. Taste-related side effects were infrequent at all dose levels with taste alteration observed in 4.8%, 6.5% and 4.8% of participants at 12.5 mg BID, 50 mg BID and 200 mg BID, respectively. No participant reported complete or partial taste loss and there were no discontinuations due to taste-related adverse events.

The treatment emergent adverse event profile was comparable to placebo. There were no treatment emergent serious adverse events reported in the trial.

Next Steps:

The Company intends to request an End of Phase 2 meeting with the FDA that is expected to take place in 2Q 2022 to discuss the Phase 3 program which is expected to start in 2H 2022.

"With no specific treatments approved for refractory chronic cough, patients and physicians struggle to manage this condition that significantly impacts the quality of life of those afflicted. The SOOTHE trial topline results generated from this large, multicenter, controlled trial are truly exciting because the impressive efficacy and tolerability profile observed for BLU-5937 could significantly benefit refractory chronic cough patients," said Dr. Jaclyn Smith, Professor of Respiratory Medicine at the University of Manchester in the United Kingdom and an Honorary Consultant at the University Hospital of South Manchester NHS Foundation Trust, and Principal Investigator of the Phase 2b SOOTHE trial.

Dr. Catherine Bonuccelli, Chief Medical Officer of BELLUS Health added, "The Phase 2b SOOTHE topline results demonstrated an important treatment benefit of BLU-5937 and its potentially best-in-class tolerability profile, with low taste-related side effects observed. We look forward to incorporating the SOOTHE results into our accelerated planning for Phase 3 with the objective of bringing this treatment option to patients with refractory chronic cough as efficiently as possible."

Update on P2X3 Pipeline

Summary of Topline Results: Phase 2a Proof-of-Concept BLUEPRINT Trial in Chronic Pruritus

In the Phase 2a proof-of-concept BLUEPRINT trial in patients with chronic pruritus associated with atopic dermatitis (“AD”), BLU-5937 (200 mg BID) did not achieve statistical significance for the primary endpoint of placebo-adjusted reduction in weekly mean Worst Itch-Numeric Rating Scale (“WI-NRS”). BLU-5937 was well-tolerated and the treatment emergent adverse event profile was comparable to placebo. The Company does not intend to further pursue development of BLU-5937 in pruritic conditions.

P2X3 Pipeline

The success of the Phase 2b SOOTHE trial further validates the role of P2X3 in cough hypersensitivity. The Company intends to evaluate potential opportunities to study BLU-5937 in additional cough indications where cough hypersensitivity plays an important role.

About SOOTHE

The SOOTHE trial is a multicenter, randomized, double-blind, four-week, parallel arm, placebo-controlled Phase 2b trial evaluating three doses of BLU-5937 (12.5 mg, 50 mg and 200 mg BID) in 310 participants with RCC. A total of 249 participants with a baseline awake cough frequency of ≥25 awake coughs per hour were randomized across four arms (1:1:1:1) evaluating the three active doses of BLU-5937 and placebo in the main study. Treatment arms were stratified to balance the number of participants with baseline awake cough frequency ≥45 coughs per hour across trial arms. The primary efficacy endpoint is the placebo-adjusted change in the 24-hour cough frequency from baseline to day 28 collected with a cough recorder. An exploratory group of an additional 61 participants with a baseline awake cough frequency of ≥10 and <25 coughs per hour were randomized across 2 arms (1:1) evaluating one active dose (200 mg BID) and placebo to further investigate the effect of BLU-5937 in patients with lower cough frequency. More information about the trial is available at www.clinicaltrials.gov: NCT04678206.

About BLUEPRINT

The BLUEPRINT trial is a multicenter, randomized, double-blind, placebo-controlled, parallel-design Phase 2a trial evaluating the efficacy, safety and tolerability of BLU-5937 in chronic pruritus associated with AD. More information about the trial is available at www.clinicaltrials.gov: NCT04693195.

Conference Call & Webcast Information:

The Company will host a conference call and webcast to discuss the topline results from the Phase 2b SOOTHE trial on December 13, 2021 at 8:00 a.m. ET.

Individuals can participate in the conference call by dialing 877-405-1224 (domestic) or 201-389-0848 (international) and referring to the “BELLUS Phase 2b SOOTHE Trial Topline Results.” The live webcast of the event may be accessed through the Events and Presentations page of BELLUS Health’s website, under the Investors & News section.

The archived webcast will be available for replay on the BELLUS Health website after the event.

About BELLUS Health (www.bellushealth.com)

BELLUS Health is a clinical-stage biopharmaceutical company developing novel therapeutics for the treatment of RCC and other hypersensitization-related disorders. The Company's product candidate, BLU-5937, has successfully completed a Phase 2b trial in RCC and is planning a Phase 3 program that is expected to begin in 2022.

RCC is a cough lasting more than 8 weeks despite appropriate treatment for underlying condition(s). It is estimated that there are approximately 9 million patients in the United States suffering from RCC. RCC is associated with significant adverse physical, social, and psychosocial effects on health and quality of life. Currently, there is no specific therapy approved for RCC and treatment options are limited.

The Company is exploring the potential use of BLU-5937 in other patient populations experiencing cough hypersensitivity as well as other P2X3-related hypersensitization conditions.

Forward-Looking Statements

Certain statements contained in this news release, other than statements of fact that are independently verifiable at the date hereof, may constitute "forward-looking statements" within the meaning of Canadian securities legislation and regulations, the U.S. Private Securities Litigation Reform Act of 1995, as amended, and other applicable securities laws. Forward-looking statements are frequently, but not always, identified by words such as “expects,” “anticipates,” “believes,” “intends,” “estimates,” “potential,” “possible,” “projects,” “plans,” and similar expressions. Such statements, based as they are on the current expectations of management, inherently involve numerous important risks, uncertainties and assumptions, known and unknown, many of which are beyond BELLUS Health's control. Such statements include, but are not limited to, the potential of BLU-5937 to successfully treat RCC and other hypersensitization-related disorders and benefit such patients, BELLUS Health’s expectations related to its preclinical studies and clinical trials, including the design, timing and results of its Phase 2b SOOTHE clinical trial of BLU-5937 in RCC, including the timing and outcome of interactions with regulatory agencies, the potential activity and tolerability profile, selectivity, potency and other characteristics of BLU-5937, including as compared to other competitor candidates, the timing of initiation of its Phase 3 clinical trial of BLU-5937 in RCC, the commercial potential of BLU-5937, including with respect to patient population, pricing and labeling, BELLUS Health’s intention to discontinue development of BLU-5937 in pruritic conditions and the Phase 2a proof-of-concept BLUEPRINT trial, BELLUS Health’s financial position, and the potential applicability of BLU-5937 and BELLUS Health’s P2X3 platform to treat other disorders. Risk factors that may affect BELLUS Health’s future results include but are not limited to: the benefits and impact on label of its enrichment strategy, estimates and projections regarding the size and opportunity of the addressable RCC market for BLU-5937, the ability to expand and develop its project pipeline, the ability to obtain adequate financing, the ability of BELLUS Health to maintain its rights to intellectual property and obtain adequate protection of future products through such intellectual property, the impact of general economic conditions, general conditions in the pharmaceutical industry, the impact of the ongoing COVID-19 pandemic on BELLUS Health’s operations, plans and prospects, including to the initiation and completion of clinical trials in a timely manner or at all, changes in the regulatory environment in the jurisdictions in which BELLUS Health does business, supply chain impacts, stock market volatility, fluctuations in costs, changes to the competitive environment due to consolidation, achievement of forecasted burn rate, potential

payments/outcomes in relation to indemnity agreements and contingent value rights , achievement of forecasted preclinical study and clinical trial milestones, reliance on third parties to conduct preclinical studies and clinical trials for BLU-5937 and that actual results may differ from topline results once the final and quality-controlled verification of data and analyses has been completed. In addition, the length of BELLUS Health's product candidate's development process and its market size and commercial value are dependent upon a number of factors. Moreover, BELLUS Health's growth and future prospects are mainly dependent on the successful development, patient tolerability, regulatory approval, commercialization and market acceptance of its product candidate BLU-5937 and other products. Consequently, actual future results and events may differ materially from the anticipated results and events expressed in the forward-looking statements. BELLUS Health believes that expectations represented by forward-looking statements are reasonable, yet there can be no assurance that such expectations will prove to be correct. The reader should not place undue reliance, if any, on any forward-looking statements included in this news release. These forward-looking statements speak only as of the date made, and BELLUS Health is under no obligation and disavows any intention to update publicly or revise such statements as a result of any new information, future event, circumstances or otherwise, unless required by applicable legislation or regulation. Please see BELLUS Health's public filings with the Canadian securities regulatory authorities, including, but not limited to, its Annual Information Form, and the United States Securities and Exchange Commission, including, but not limited to, its Annual Report on Form 40-F, for further risk factors that might affect BELLUS Health and its business.

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FOR MORE INFORMATION:
Ramzi Benamar
Chief Financial Officer
rbenamar@bellushealth.com

Media:
Brad Miles
Solebury Trout
bmiles@soleburytrout.com

Source: BELLUS Health Inc.